Biogen Receives U.S. FDA Breakthrough Therapy Designation for Felzartamab for the Treatment of Antibody-Mediated Rejection in Kidney Transplant Recipients

October 9, 2024

- Designation is based on data from the clinical development program which demonstrated clinical proof of concept
- Felzartamab, an investigational anti-CD38 monoclonal antibody, is a potential first-in-class therapeutic candidate for a range of rare immune-mediated indications with planning underway for Phase 3 development
- FDA Breakthrough Therapy Designation is intended to expedite the development and review of drugs for serious or life-threatening conditions and which have potential to show substantial improvement over existing therapies

CAMBRIDGE, Mass., Oct. 09, 2024 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) – Biogen announced today that felzartamab, an investigational anti-CD38 monoclonal antibody, has received Breakthrough Therapy Designation (BTD) from the U.S. Food and Drug Administration (FDA) for the treatment of late antibody-mediated rejection (AMR) without T-cell mediated rejection in kidney transplant patients. The FDA grants BTD to drug candidates for serious or life-threatening conditions and that have preliminary clinical evidence demonstrating potential to provide substantial improvement over existing therapies. The designation provides additional opportunities to engage the FDA and to support the drug development program through Fast Track designation features.

Data from the clinical development program that supported the designation were published in the *New England Journal of Medicine* and presented as a late-breaking presentation at the 61st European Renal Association (ERA) Congress in Stockholm, May 2024.

"Antibody-mediated rejection is a major reason why kidney transplants fail, and currently patients suffering from AMR have tremendous unmet medical need," said Travis Murdoch, Head of HI-Bio at Biogen. "We are focused on tackling this important challenge, and the breakthrough therapy designation will enable us to work efficiently with the FDA to accelerate development of felzartamab in AMR."

Felzartamab previously received BTD and Orphan Drug Designation (ODD) from the FDA for development in the treatment of primary membranous nephropathy (PMN) and ODD in the treatment of AMR in kidney transplant recipients. Phase 2 studies have been completed in AMR, PMN and IgA nephropathy (IgAN). Biogen plans to initiate Phase 3 trials for felzartamab across AMR, IgAN, and PMN in 2025.

Biogen acquired Human Immunology Biosciences (HI-Bio) in July 2024.

About Felzartamab
Felzartamab is an investigational therapeutic human monoclonal antibody directed against CD38, a protein expressed on mature plasma cells. Felzartamab has been shown in clinical studies to selectively deplete CD38+ plasma cells, which may allow applications that ultimately improve clinical outcomes in a broad range of diseases driven by pathogenic antibodies. Felzartamab was originally developed by MorphoSys AG for multiple myeloma. HI-Bio exclusively licensed the rights to develop and commercialize felzartamab across all indications in all countries and territories excluding China (including Macau and Hong Kong and Taiwan).

Felzartamab is an investigational therapeutic candidate that has not yet been approved by any regulatory authority and its safety and effectiveness have not been established.

About Antibody-Mediated Rejection (AMR) in Kidney Transplant Recipients
Antibody-mediated rejection (AMR) is a major cause of kidney transplant failure, with chronic AMR affecting ~12% of patients that receive kidney transplants annually in the U.S.[1] AMR has emerged as the leading cause of late graft loss in kidney transplant recipients. Effective treatment options for chronic AMR are currently limited.[2]

About Biogen
Founded in 1978, Biogen is a leading biotechnology company that pioneers innovative science to deliver new medicines to transform patients' lives and to create value for shareholders and our communities. We apply deep understanding of human biology and leverage different modalities to advance first-in-class treatments or therapies that deliver superior outcomes. Our approach is to take bold risks, balanced with return on investment to deliver long-term growth.

We routinely post information that may be important to investors on our website at www.biogen.com. Follow us on social media - Facebook, LinkedIn, X, YouTube.

Biogen Safe Harbor
This news release contains forward-looking statements, including related to the potential clinical effects of felzartamab; the potential benefits, safety and efficacy of felzartamab; the clinical development program for felzartamab; the identification and treatment of AMR; our research and development program for the treatment of AMR; the potential of our commercial business and pipeline programs, including SPINRAZA; and risks and uncertainties associated with drug development and commercialization. These forward-looking statements may be accompanied by words such as "aim," "anticipate," "believe," "could," "estimate," "expect," "forecast," "intend," "may," "plan," "potential," "possible," "will," "would" and other words and terms of similar meaning. Drug development and commercialization involve a high degree of risk, and only a small number of research and development programs result in commercialization of a product. Results in early-stage clinical trials may not be indicative of full results or results from later stage or larger scale clinical trials and do not ensure regulatory approval. You should not place undue reliance on our forward-looking statements.

These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements, including without limitation, uncertainty of success in the development and potential commercialization of SPINRAZA; the risk that we may not fully enroll our clinical trials or enrollment will take longer than expected; unexpected concerns may arise from additional data, analysis or results obtained during our clinical trials; regulatory authorities may require additional information or further studies, or may fail or refuse to approve or may delay approval of our drug candidates, including SPINRAZA; the occurrence of adverse safety events; the risks of unexpected hurdles, costs or delays; failure to protect and enforce our data, intellectual property and other proprietary rights and uncertainties relating to intellectual property claims and challenges; product

liability claims; results of operations and financial condition. The foregoing sets forth many, but not all, of the factors that could cause actual results to differ from our expectations in any forward-looking statement. Investors should consider this cautionary statement, as well as the risk factors identified in our most recent annual or quarterly report and in other reports we have filed with the U.S. Securities and Exchange Commission. These statements speak only as of the date of this news release.

We do not undertake any obligation to publicly update any forward-looking statements.

References:

1. Schinstock et al. (2018) Kidney Transplant with Low Levels of DSA or Low Positive B-Flow Crossmatch: An Underappreciated Option for Highly-Sensitized Transplant Candidates (Page 8). Available at: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5481511/pdf/nihms837168.pdf#page=8 ; Ciancio et al. 2018 Antibody-Mediated Rejection Implies a Poor Prognosis in Kidney Transplantation: Results From a Single Center. Available at: https://onlinelibrary.wiley.com/doi/10.1111/ctr.13392
2. Rodriguez-Ramirez et al. 2022 Antibody-mediated rejection: prevention, monitoring and treatment dilemmas (Page 1). Available at: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC9475491/

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